FNAIT Systematic Literature Review and Meta-Analysis Presented at the Academy of Managed Care Pharmacy 2024 Annual Meeting

April 17, 2024 at 4:01 PM EDT

- Data Support HPA-1a Negative Frequency of More than 2% in Nearly 200,000 Screened Pregnant Women -

- Among HPA-1a Negative Pregnant Women, Approximately 33% are at Higher Risk for Alloimmunization -

NEW HAVEN, Conn.--(BUSINESS WIRE)--Apr. 17, 2024-- Rallybio Corporation (Nasdaq: RLYB), a clinical-stage biotechnology company committed to identifying and accelerating the development of life-transforming therapies for patients with severe and rare diseases, today announced the presentation of results from a fetal and neonatal alloimmune thrombocytopenia (FNAIT) systematic literature review and meta-analysis at the Academy of Managed Care Pharmacy (AMCP) 2024 Annual Meeting, which is taking place in New Orleans, LA. The results of this research found that, in a pooled analysis of 198,062 pregnant women, 2.2% were HPA-1a negative and 32.3% of these women were also HLA-DRB3*01:01 positive and therefore at ~25-fold higher risk for alloimmunization. These rates are consistent with Rallybio's current estimate of annual at-risk pregnancies and translate to tens of thousands of fetuses and newborns at risk each year for the potentially devastating consequences of FNAIT.

"We are pleased to establish a robust foundation of knowledge documenting the frequency of FNAIT risk as reported from a pooled analysis of peer-review literature, which is consistent with our current estimates," said Stephen Uden, M.D., Chief Executive Officer of Rallybio. "This information, in combination with data from our ongoing FNAIT natural history study, will enable us to create a shared understanding of the number of pregnant women and babies at higher risk of FNAIT annually, underscoring the importance of having an effective preventative therapeutic option."

Rallybio is developing RLYB212, a novel human monoclonal anti-HPA-1a antibody designed to prevent alloimmunization in pregnant women, thereby eliminating the risk of FNAIT and its potentially devastating consequences in their fetuses and newborns. Rallybio is on track to initiate a Phase 2 dose confirmation study for RLYB212 in pregnant women in the second half of 2024. The company is also conducting an ongoing FNAIT natural history study that will provide a contemporary dataset for HPA-1a alloimmunization frequency in a racially and ethnically diverse population, which is intended to support a future Phase 3 registration study for RLYB212. RLYB212 is the only investigational therapy currently reported to be in clinical development to address the needs of pregnant women at higher risk of FNAIT who have not alloimmunized.

The poster, titled "Fetal and Neonatal Alloimmune Thrombocytopenia: A Systematic Literature Review and Meta-analysis of Adverse Pregnancy-Related Outcomes to Support the Development of a Novel Prophylactic Therapeutic," was presented by Andrea V. Margulis of RTI Health Solutions. Specifically, the literature review found that, of 198,062 screened pregnant women, 2.2% (95% confidence interval [CI], 2.0%-2.5%) were HPA-1a negative; 32.3% (28.6%-36.1%) of HPA-1a–negative women were HLA-DRB3*01:01 positive and therefore at even higher risk for alloimmunization. Approximately 10% of HPA-1a–negative women were already alloimmunized to HPA-1a. The meta-analysis is based on 12 observational cohort studies from Europe, Canada, and Egypt published from 1985 through 2018. A link to the poster is available here.

About FNAIT

Fetal and Neonatal Alloimmune Thrombocytopenia (FNAIT) is a potentially life-threatening rare disease that can cause uncontrolled bleeding in fetuses and newborns. FNAIT can arise during pregnancy due to an immune incompatibility between an expectant mother and her fetus in a specific platelet antigen called human platelet antigen 1, or HPA-1.

There are two predominant forms of HPA-1, known as HPA-1a and HPA-1b, which are expressed on the surface of platelets. Individuals who are homozygous for HPA-1b, meaning that they have two copies of the HPA-1b allele and no copies of the HPA-1a allele, are also known as HPA-1a negative. Upon exposure to the HPA-1a antigen, these individuals can develop antibodies to that antigen in a process known as alloimmunization. In HPA-1a-negative expectant mothers bearing a HPA-1a-positive fetus, alloimmunization can occur upon mixing of fetal blood with maternal blood. When alloimmunization occurs in an expectant mother, the anti-HPA-1a antibodies that develop in the mother can cross the placenta and destroy platelets in the fetus. The destruction of platelets in the fetus can result in severely low platelet counts, or thrombocytopenia, and potentially lead to devastating consequences including miscarriage, stillbirth, death of the newborn, or severe lifelong neurological disability in those babies who survive. There is currently no approved therapy for the prevention or prenatal treatment of FNAIT.

About Rallybio

Rallybio (Nasdaq: RLYB) is a clinical-stage biotechnology company with a mission to develop and commercialize life-transforming therapies for patients with severe and rare diseases. Rallybio has built a broad pipeline of promising product candidates aimed at addressing diseases with unmet medical need in areas of maternal fetal health, complement dysregulation, hematology, and metabolic disorders. The Company has two clinical stage programs: RLYB212, an anti-HPA-1a antibody for the prevention of fetal and neonatal alloimmune thrombocytopenia (FNAIT) and RLYB116, an inhibitor of complement component 5 (C5), with the potential to treat several diseases of complement dysregulation, as well as additional programs in preclinical development. Rallybio is headquartered in New Haven, Connecticut. For more information, please visit www.rallybio.com and follow us on LinkedIn and Twitter.

Forward-Looking Statements

This press release contains forward-looking statements that are based on our management's beliefs and assumptions and on currently available information. All statements, other than statements of historical facts contained in this press release are forward-looking statements. In some cases, forward-looking statements can be identified by terms such as "may," "will," "should," "expect," "plan," "anticipate," "could," "intend," "target," "project," "contemplate," "believe," "estimate," "predict," "potential" or "continue" or the negative of these terms or other similar expressions, although not all forward-looking statements contain these words. Forward-looking statements in this press release include, but are not limited to, statements

concerning the rates of women who are HPA-1a negative and HLA-DRB3*01:01 positive, the level of increased higher risk for alloimmunization among women who are HPA-1a negative and HLA-DRB3*01:01 positive, the actual rates of annual at-risk pregnancies for FNAIT and the number of potential pregnancies that could be at risk, our belief that the literature review establishes the relevant foundation of knowledge regarding such rates, our ability to create a shared understanding of the number of pregnant women and babies at higher risk of FNAIT annually, the timing of initiation of the Phase 2 dose confirmation study for RLYB212, whether the results of the natural history study and the planned Phase 2 dose confirmation study will be sufficient to support design and implementation of a Phase 3 registrational study for RLYB212, and the likelihood that Rallybio will be successful in developing RLYB212. The forward-looking statements in this press release are only predictions and are based largely on management's current expectations and projections about future events and financial trends that management believes may affect Rallybio's business, financial condition, and results of operations. These forward-looking statements speak only as of the date of this press release and are subject to a number of known and unknown risks, uncertainties and assumptions, including, but not limited to, our ability to successfully initiate and conduct our planned clinical studies, and complete such clinical studies and obtain results on our expected timelines, or at all, whether our cash resources will be sufficient to fund our operating expenses and capital expenditure requirements and whether we will be successful raising additional capital, our ability to enter into strategic partnerships or other arrangements, competition from other biotechnology and pharmaceutical companies, and those risks and uncertainties described in Rallybio's filings with the U.S. Securities and Exchange Commission (SEC), including Rallybio's Annual Report on Form 10-K for the period ended December 31, 2023, and subsequent filings with the SEC. The events and circumstances reflected in our forward-looking statements may not be achieved or occur and actual future results, levels of activity, performance and events and circumstances could differ materially from those projected in the forward-looking statements. Except as required by applicable law, we are not obligated to publicly update or revise any forward-looking statements contained in this press release, whether as a result of any new information, future events, changed circumstances or otherwise.

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